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Title: Applicability of Existing Regulations to the Development of a Dendrimer Nanotechnology-Based Pharmaceutical

Summary:

This presentation will provide an overview of the development of a dendrimer nanotechnology-based pharmaceutical in the context of existing regulations.

The purpose of regulation is to ensure the benefits and risks of products to the public are balanced. Successful regulation of nanotechnology as a broad concept would rely on nanotechnology being a readily and consistently identifiable and definable technology. Due to widespread designation of a range of products or technologies as 'nanotechnology', a single, overarching definition of what constitutes nanotechnology is difficult to establish. As a result, specific regulation of non-specific products and technologies will be a challenge for those developing, marketing and regulating nanotechnologies.

Dendrimers are a class of molecule being developed for a range of commercial applications including as stand-alone pharmaceuticals and drug delivery agents. Dendrimers are synthesized through controlled, manufacturing processes, not entirely dissimilar to the chemistry of small molecules. Because of the size of the dendrimer in the nanometer scale, there are also some similarities with the manufacturing processes of large biomolecules.

In contrast to small molecules, dendrimers have many surface attachment points, to which small molecules can be attached. When attached to the dendrimer, the multiple presentation of some small molecules results in novel pharmaceutical properties when compared with simple presentation of the small molecules alone.

Dendrimers may also be designed to carry intact, active drugs to a target organ or tissue, where the active could be released and elicit a therapeutic response.

In contrast to large biomolecules, dendrimers have a very defined structure based on the chemistry of the components used to construct the molecule.

Therefore, dendrimers are a unique form of nanotechnology.

This presentation will discuss some of the challenges encountered in the development of a dendrimer nanotechnology-based pharmaceutical, thought to be the first dendrimer product of its kind reviewed by FDA, and will highlight the applicability of existing regulations to this process.

A list of the key issues to be discussed in the presentation follows:

- Drug/device considerations of dendrimer products
- Meeting the challenges of manufacturing and characterization of nanostructures
- Investigating the fate of a dendrimer and its metabolites or components
- Successfully assessing safety and efficacy of nanotechnology products
- Advancing to clinical development of dendrimers
- Negotiating regulatory agency review, and engaging the regulator
- Addressing environmental impact
- Implementing protections in the workplace
- Meeting the needs of key interest groups

To the current stage of development, existing regulations have been found to adequately address all aspects of development of dendrimer nanotechnology to ensure the development of a sound pharmaceutical candidate and the wellbeing of the public.

Into the foreseeable future, it appears that existing regulations will also adequately address development of dendrimer nanotechnologies as pharmaceuticals and drug delivery agents to the point of marketing and beyond.

Specific regulation may be required for some classes of nanotechnology. Regulation should be applied to products, on a case by case basis, not to a technology as a whole.

Caution should be employed to ensure that new regulations, if required, do not restrict the development of new beneficial technologies, do not impact other technology developments outside of the field of nanotechnology, and uphold the principle of ensuring the balance between risk and benefit for the public.

End.